
"Stem Cell Therapies for Heart Failure"

Grant Award Details

"Stem Cell Therapies for Heart Failure"

Grant Type: Disease Team Planning

Grant Number: DT1-00704

Investigator:

Name:	Mark Mercola
Institution:	Sanford-Burnham Medical Research Institute
Type:	PI

Disease Focus: Heart Disease, Heart failure

Award Value: \$44.450

Status: Closed

Grant Application Details

Application Title: "Stem Cell Therapies for Heart Failure"

Public Abstract:

Our multi-institutional program is dedicated to the treatment of heart failure, which has become a leading cause of death in California and the U. S. The high death toll continues to climb despite many significant recent advances in the medical treatment of heart failure. The disease is due to a debilitating loss and/or dysfunction of heart muscle cells (cardiomyocytes) in the injured heart. Most current therapies and clinical trials are not designed to regenerate these cells. Rather, they focus on boosting output of the failing heart and/or forestalling further decline. Our intention is to regenerate or replace the damaged cardiomyocytes and their supporting cells.

Our consortium unites over 20 highly successful laboratories with expertise in cardiomyocyte stem cell biology, high throughput chemical library screening and drug discovery, biomaterials and bioengineering, cardiac imaging, heart failure therapeutics, and advanced clinical interventional cardiology. These groups will be organized into Basic Discovery, Translational, and Applied and Clinical research teams to bring two types of therapies from the research lab to the clinic. The first will be to replace damaged cardiomyocytes by transplantation of stem cell derived cardiomyocytes, either from embryonic stem cells (ESCs) or cardiomyogenic cells derived from the patient. The second will be to use stem and progenitor cells to develop drugs capable of stimulating regeneration in a patient's heart from his or her own cells. It is likely that a successful therapy will combine these two approaches. Importantly, we will develop technology to deliver stem cell-derived cells to the heart in human patients and evaluate their survival and effects on function.

The PI and lead investigators represent a multidisciplinary team consisting of basic research and clinical faculty, a number of whom hold chair, division chief or directorial positions in their institutions and have experience leading multidisciplinary projects. During the planning period, the team will carry out the following tasks:

- Develop a comprehensive research plan, including specific aims to be jointly carried out and milestones to be achieved.
- Develop a detailed plan for translating the basic findings to clinical and pharmaceutical environment that will address, in addition to scientific and medical issues, key financial, philanthropic, and legal hurdles.
- Coordinate our clinical, pharmaceutical and device industry partners to speed along translation to clinic and pharmaceutical or biotech environment.
- Coordinate management and fundraising initiatives and resolve intellectual property and contractual issues among the participating institutions
- Partially compensate a chief operating officer to coordinate the activities of the consortium.

Statement of Benefit to California: Benefits will accrue to California through:

1. California heart disease patients will benefit from improved therapies. The numbers are staggering: Of the 60 million Americans who have cardiovascular disease, 5 million have heart failure [American Heart Association (AHA) Annual Report, <http://www.americanheart.org>]. In California, it is the most common diagnosis at hospital discharge for people over age 65 and the largest cause of mortality and morbidity, with an approximately 50% survival rate after 5 years. The cost estimates to treat heart failure vary widely but are considerable, ranging from \$8 billion to over \$400 billion annually in the US (AHA estimate is about \$20 billion). According to the California HealthCare Foundation, the annual per capita cost for treatment in the state is \$13,000 (Improving Quality of Care for Californians with Heart Failure, CHCF publication, 2002). Current therapies address heart function largely through pharmaceutical management (e.g. ACE inhibitors, diuretics, inotropic agents) but are not designed to replace or supplant the muscle cells that are lost or dysfunctional. The goals of our research program will address the underlying disease and thus have potential for alleviating pain and suffering of the patient population and their families as well as decrease the financial burden to the patients' families, private insurers and state agencies.
2. Technology transfer to California institutions. Each of the participating institutions has seen and implemented a steady increase in technology transfer in the past decade. Based on these precedents, and the translational potential of our research goals, this program is likely to result in licensing of further technology to the corporate sector. This will have an impact on boosting the competitiveness of our state's technology sector with the accompanying potential for creation of new jobs.
3. Enhanced ability of California institutions to recruit stem cell scientists. Already, we have seen a number of recruits from students to senior faculty as a result and this is likely to be enhanced by additional funding. Moreover, because of the translational nature of the research and resulting technology transfer to industry partners, the proposed disease-oriented research should have a similar impact on our biotechnology and/or pharmaceutical partners.

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